Amylyx Pharmaceuticals Receives Negative CHMP Opinion on its Marketing Authorisation Application for AMX0035 for the Treatment of ALS in the European Union Following Re-Examination Process

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- PHOENIX Phase 3 trial topline results on track for mid-2024 and will inform regulatory next steps in the EU
- Final decision from the European Commission expected by the end of 2023

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 13, 2023-- Amylyx Pharmaceuticals, Inc. (NASDAQ: AMLX) ("Amylyx" or the "Company") today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) confirmed its initial negative opinion on the Marketing Authorisation Application (MAA) for AMX0035 (sodium phenylbutyrate and ursodoxicoltaurine [also known as taurursodiol]), under the trade name ALBRIOZA®, for the treatment of amyotrophic lateral sclerosis (ALS) in the European Union (EU). The decision follows the conclusion of the CHMP's formal re-examination procedure of an initial negative opinion adopted in June 2023.

ALBRIOZA was approved with conditions by Health Canada in June 2022 and granted a full approval by the U.S. Food and Drug Administration (FDA) under the trade name RELYVRIO® in September 2022.

The European Organization for Professionals and Patients with ALS (EUpALS) Patients and Carers Expert Board said in a statement, "We are disappointed to learn of this outcome, as it is a further setback for the more than 30,000 people living with ALS and their loved ones in Europe who have not seen therapeutic progress for this devastating disease in over 25 years."

"We share the frustration felt by the European ALS community, who has no time to wait for new, safe, and effective treatment options," said Stéphanie Hoffmann-Gendebien, General Manager and Head of EMEA at Amylyx. "We remain committed to exploring all potential paths forward in support of the Company's goal of getting AMX0035 to people living with ALS in the EU as quickly as possible."

"AMX0035 is the first and only drug to show an effect on both function and survival in the same trial. Since the medication's approval with conditions in Canada and full approval in the U.S., thousands of people have been prescribed AMX0035 in North America. ALS has no geographical boundaries, and we are working with urgency toward providing timely, broad, and sustainable access to AMX0035 for eligible people living with ALS who may benefit," said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx.

Amylyx continues to focus on the completion of the PHOENIX Phase 3 clinical trial, which was initiated prior to its Marketing Authorisation Application submission and will provide additional data on the efficacy and safety profile of ALBRIOZA in people living with ALS. If PHOENIX is supportive, Amylyx plans to seek approval in the EU as quickly as possible. Topline results are anticipated in mid-2024.

PHOENIX is a 48-week, randomized placebo-controlled global Phase 3 clinical trial further evaluating the safety and efficacy of AMX0035 for the treatment of ALS. The study enrolled 664 participants living with ALS across 69 sites in either Europe or the U.S., the majority of which are members of the NEALS or TRICALS consortia. The design of PHOENIX was informed by the results of the Phase 2 CENTAUR clinical trial of AMX0035, which met its prespecified primary outcome and demonstrated a statistically significant benefit in function as well as an observed benefit on survival in a longer-term post hoc analysis. Overall, reported rates of adverse events and discontinuations in CENTAUR were similar between AMX0035 and placebo groups during the 24-week randomized phase; however, gastrointestinal events occurred with greater frequency (≥2%) in the AMX0035 group. More information on the PHOENIX trial can be found at https://classic.clinicaltrials.gov/ct2/show/NCT05021536 or https://www.clinicaltrialsregister.eu/ctr-search/search, EudraCT Number: 2021-000250-26.

The CHMP opinion will be forwarded to the European Commission, who will adopt the final decision on this application. A decision is anticipated by the end of 2023.